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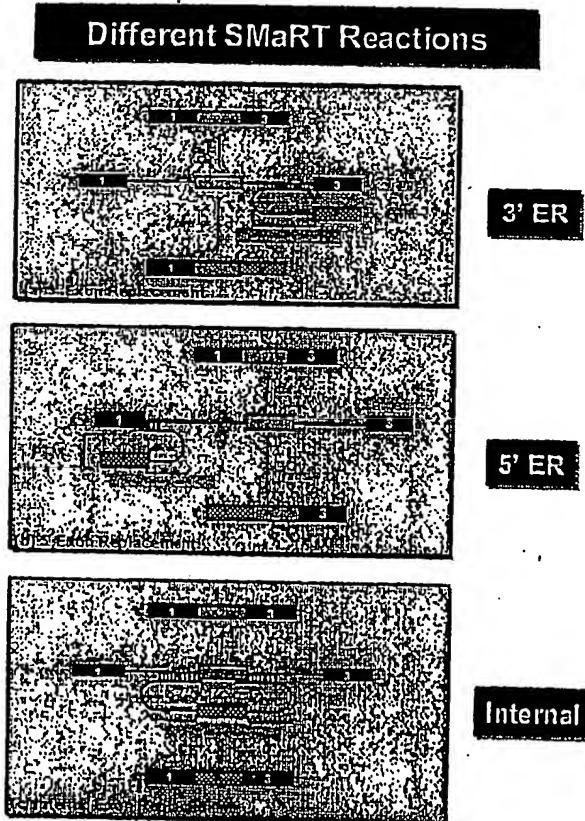
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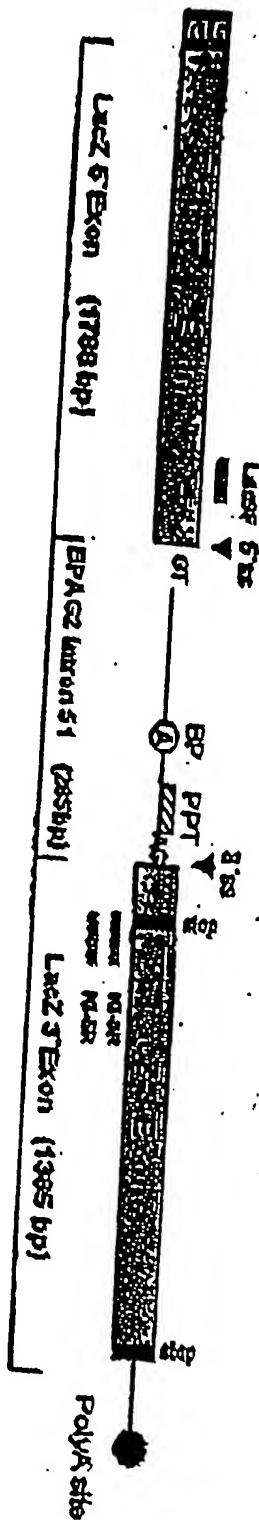
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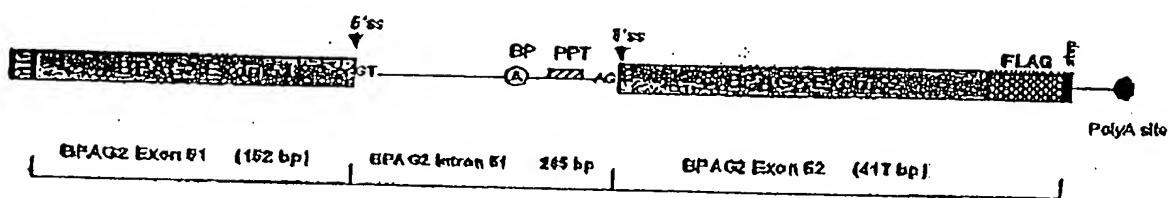
## FIGURE 1



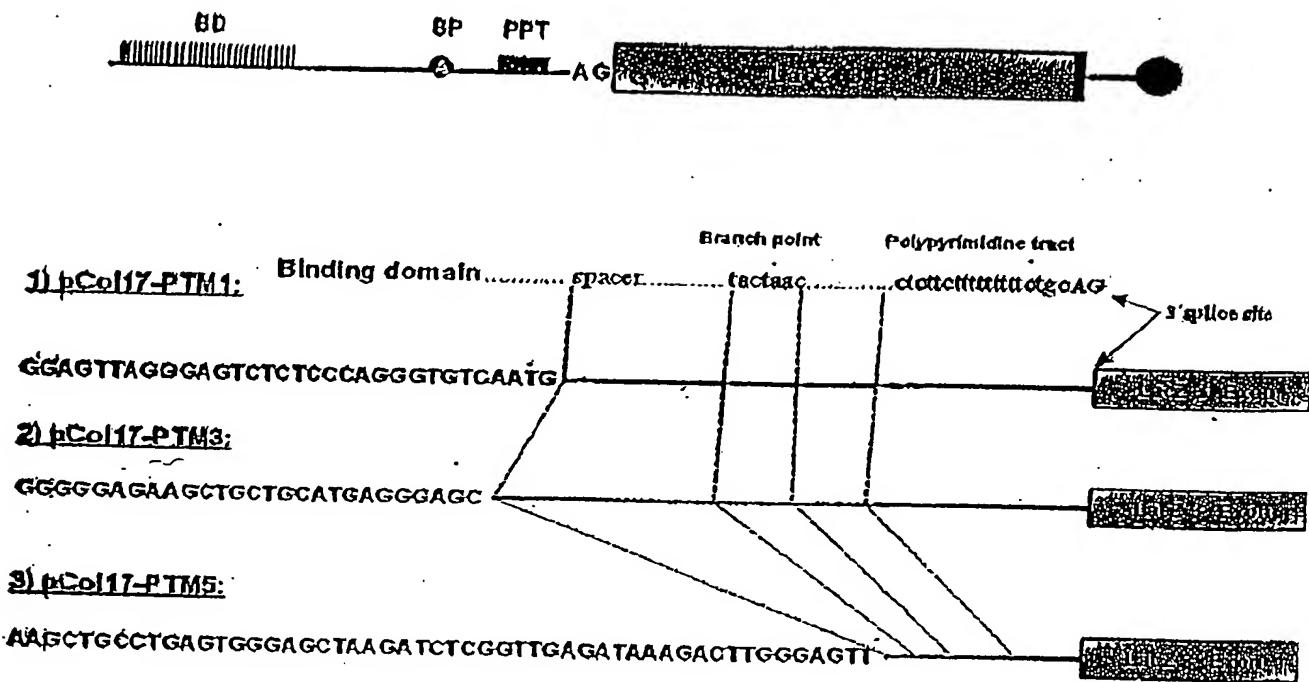
# FIGURE 2A



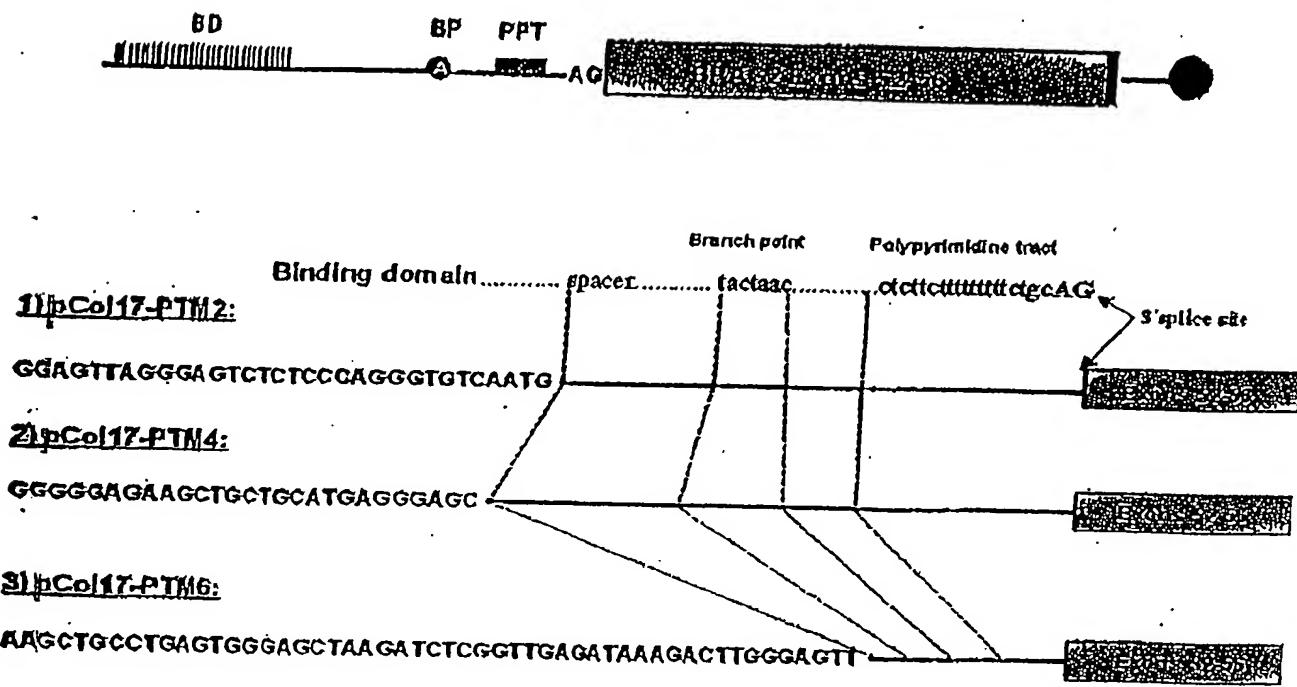
2) EPAG2 Target 2 (T2)



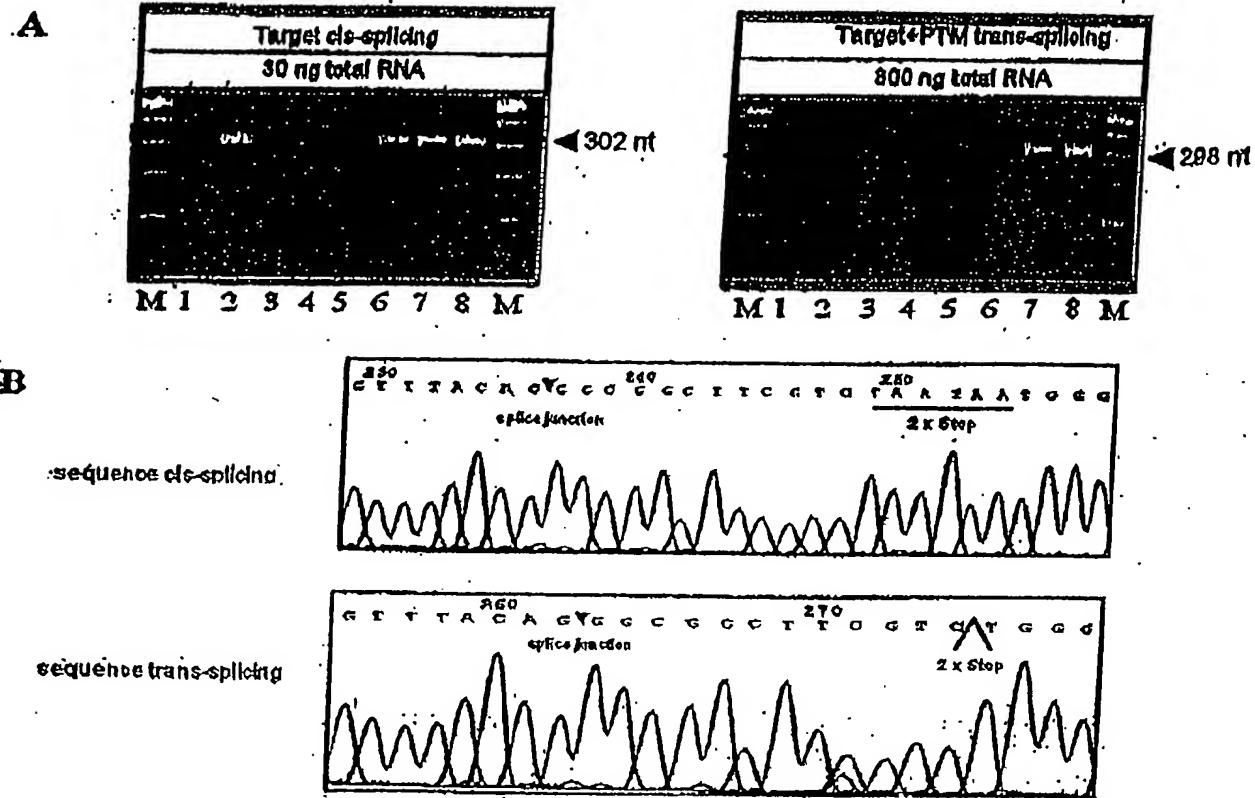
**FIGURE 2B**



## FIGURE 2C



## FIGURE 2D



**FIGURE 3A-B**

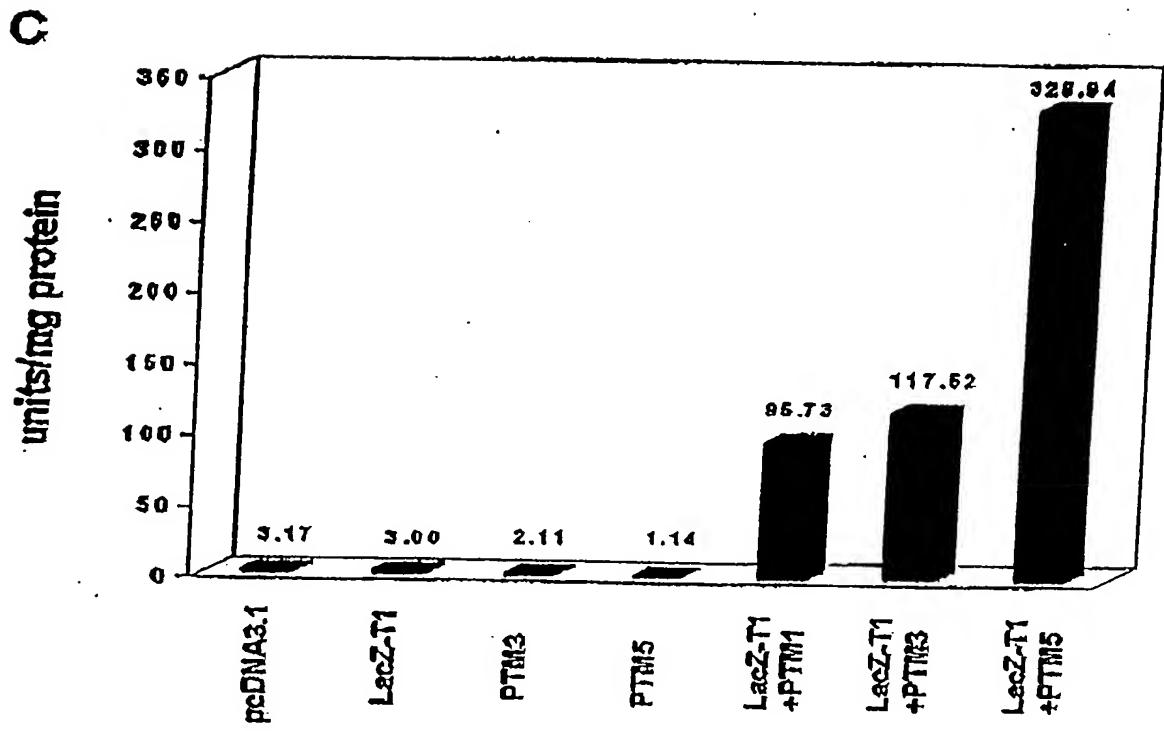


FIGURE 3C

GABEB

hKC

293T

Control  
LacZ-T1+PTM 5

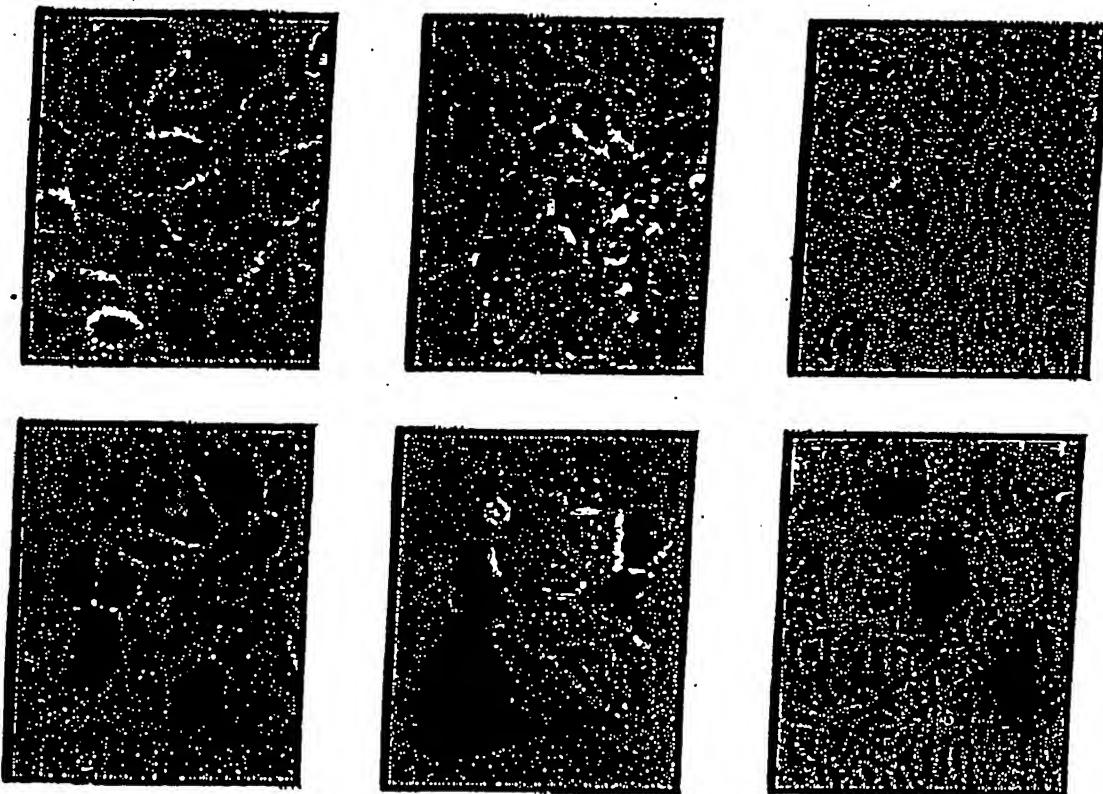
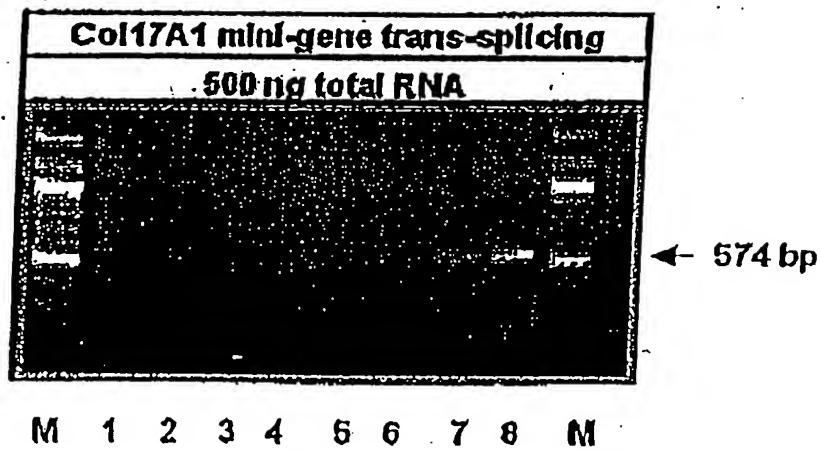
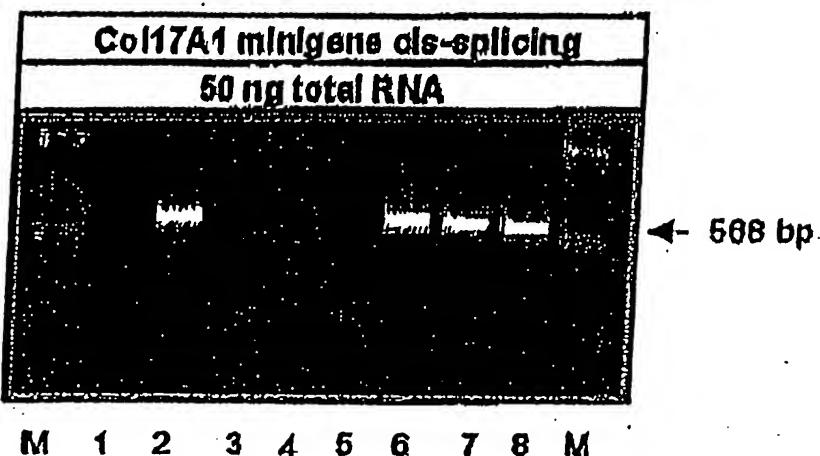


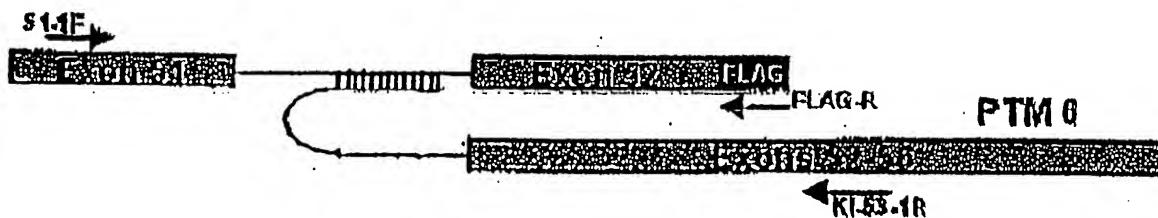
FIGURE 4

A

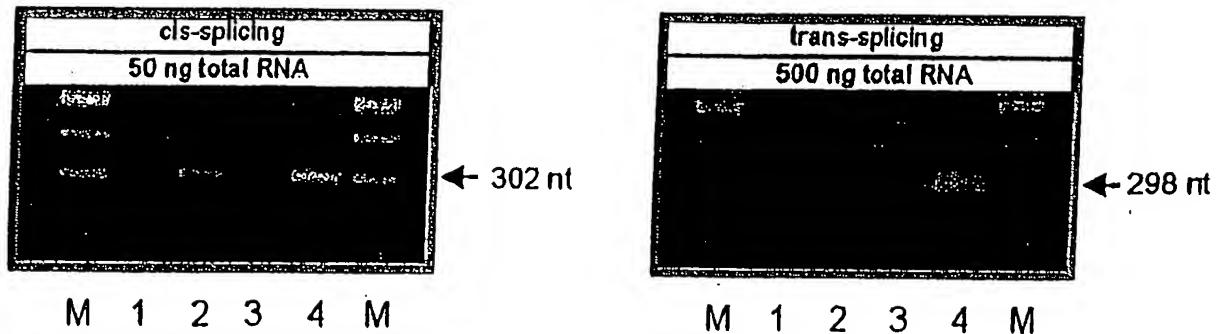
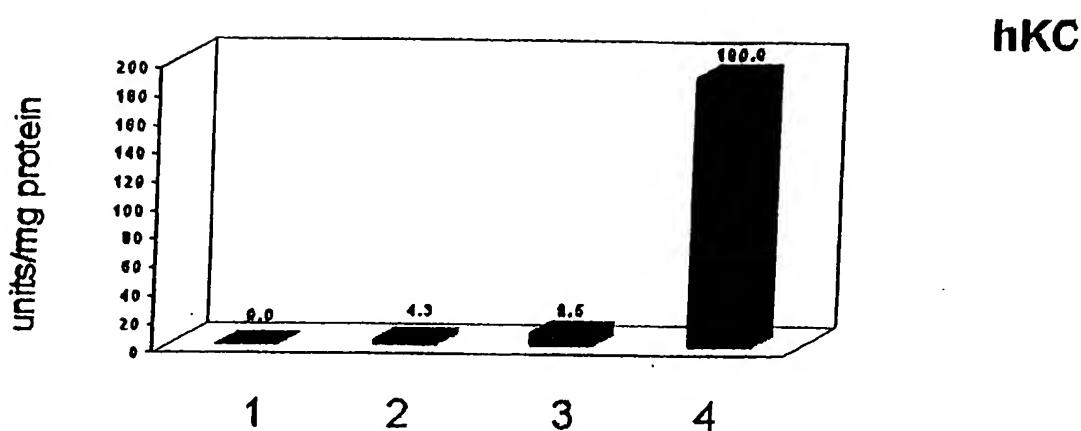


B

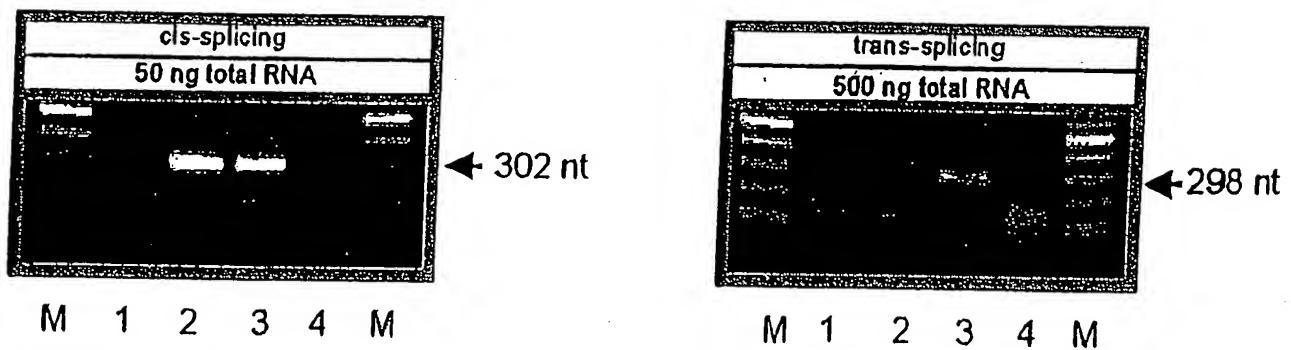
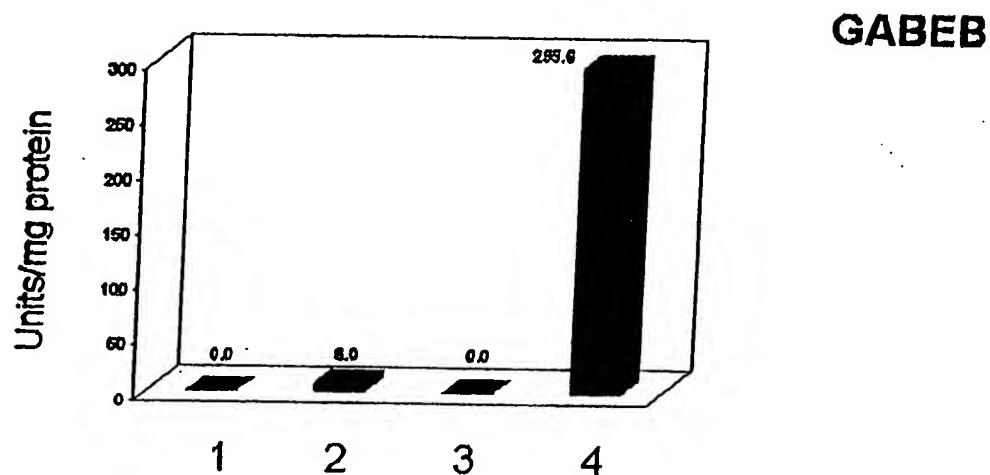
**COL17A1 minigene (T2)**



**FIGURE 5A-B**



**FIGURE 6A**



**FIGURE 6B**

# FIGURE 7

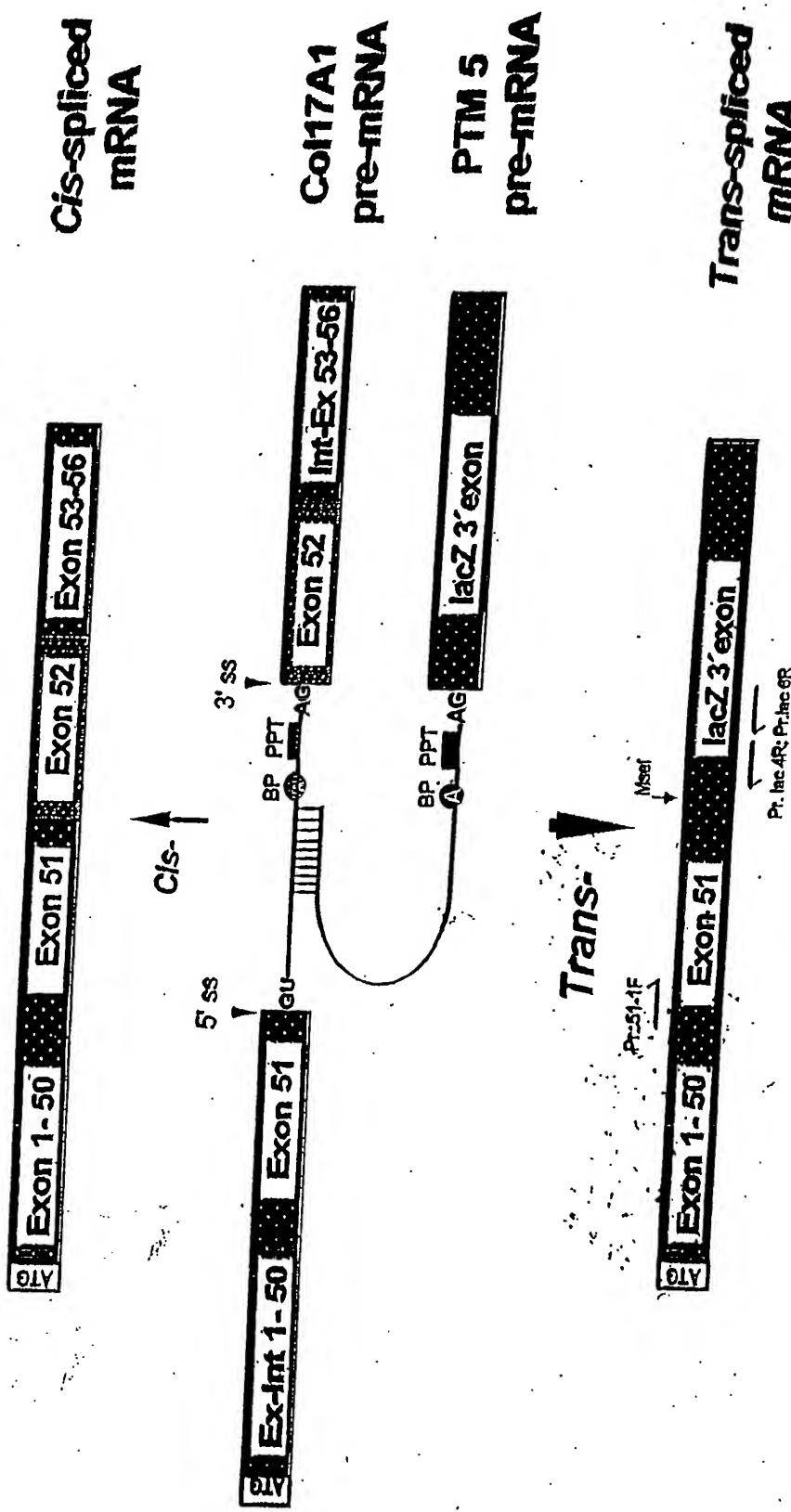
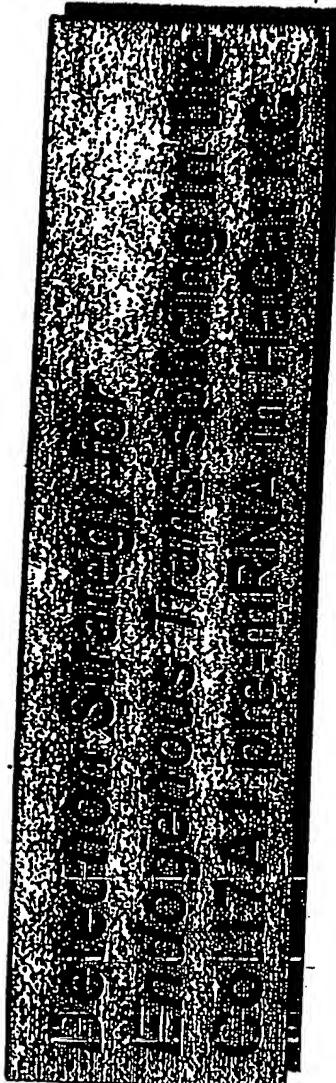
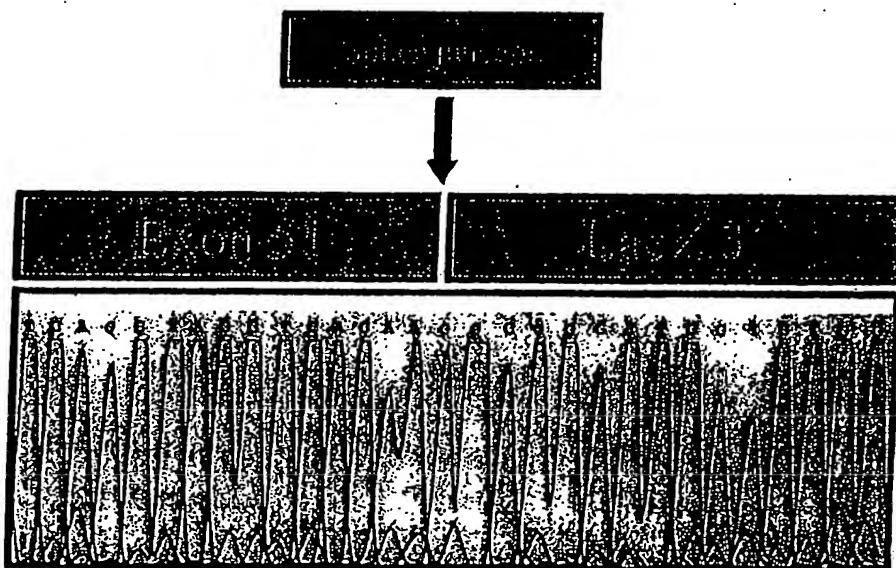


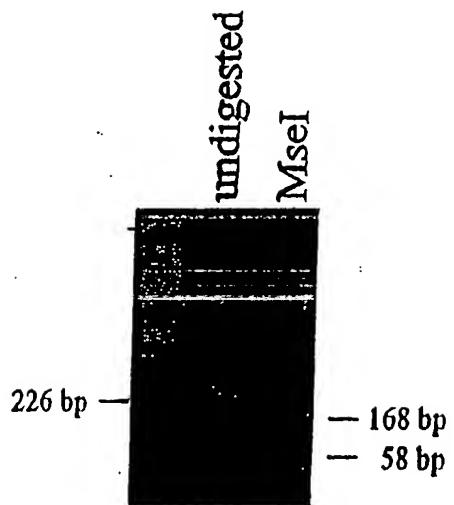
FIGURE 8

Endogenous Transsplicing  
of Col17 pre-mRNA with PTM5 pre-mRNA

A)

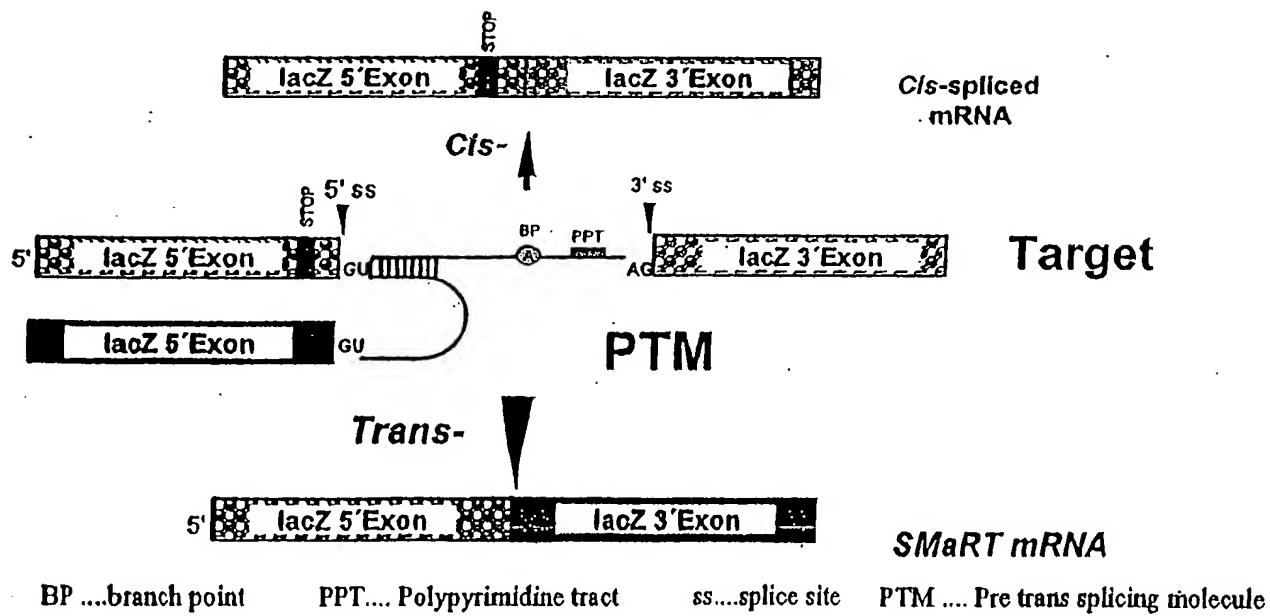


B)



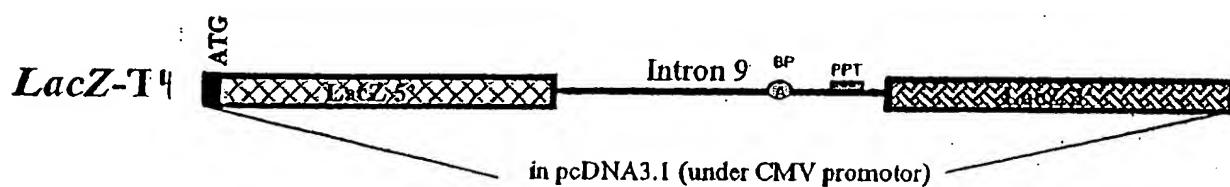
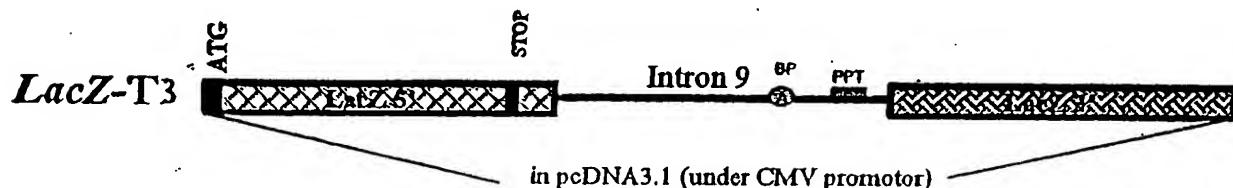
## FIGURE 9

### 5' trans-splicing in a LacZ-model system for hereditary diseases



## FIGURE 10

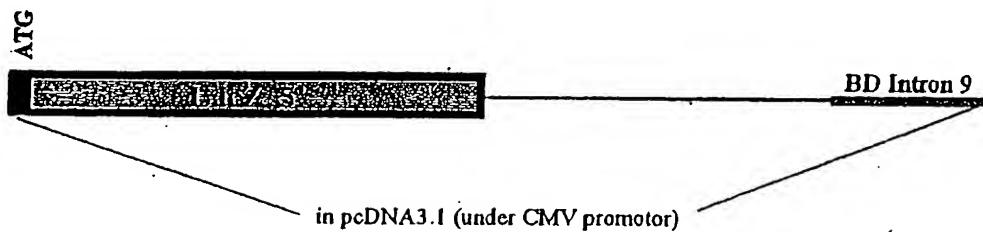
Target LacZ-T3 (containing Intron 9) and LacZ-T4 used for  
optimizing trans-splicing and transfection conditions



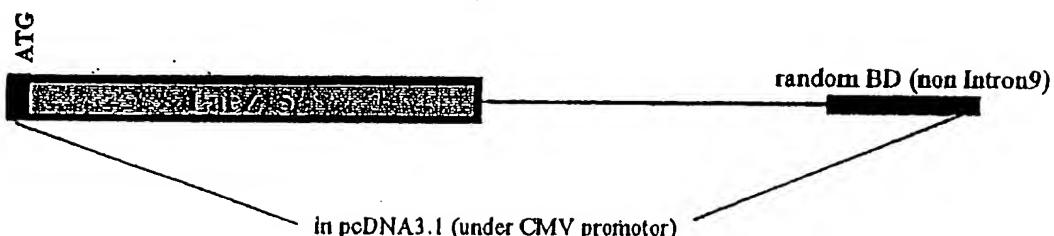
## FIGURE 11

LacZ-PTM-3 (Intron 9 specific binding domain) and LacZ-PTM-4 (non specific binding domain) for establishing optimal trans-splicing conditions

*Lac-PTM<sup>3</sup>*

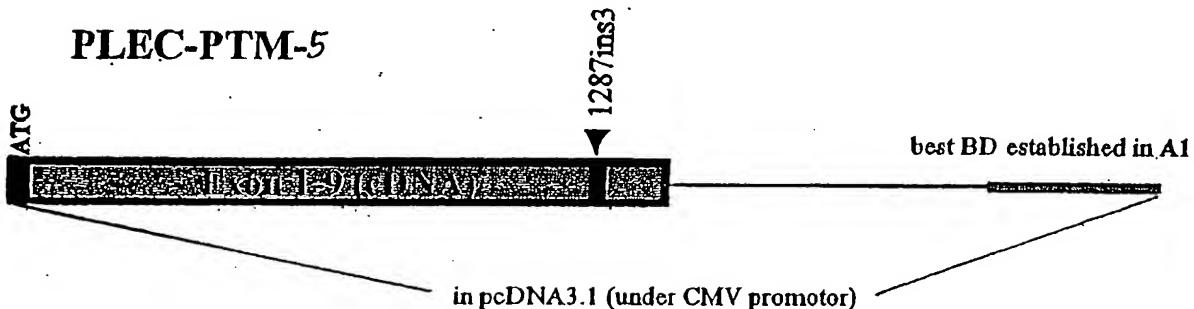


*Lac-PTM<sup>4</sup>*



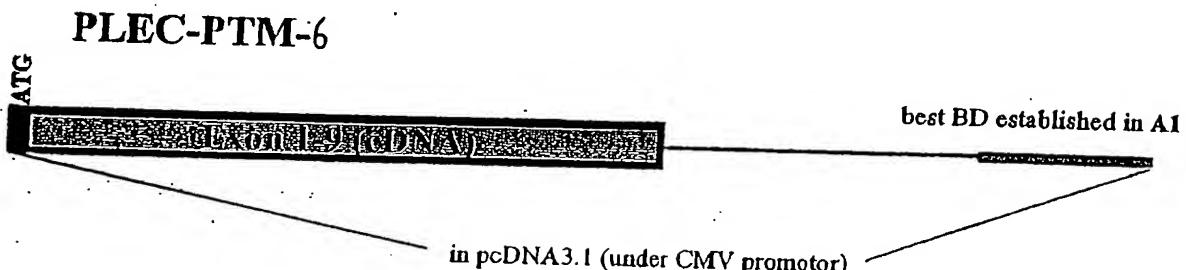
## FIGURE 12

PLEC-PTM-5 for the introduction of the 1287ins3 mutation  
in 293T cells



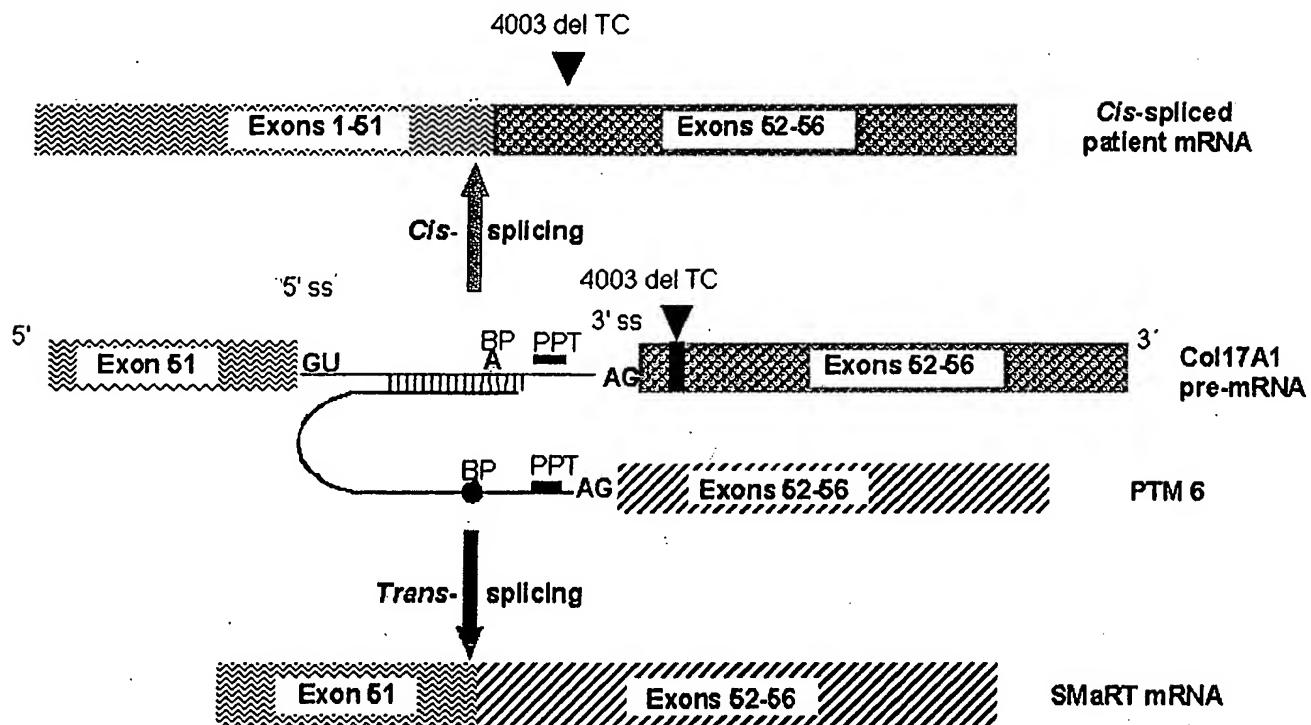
## FIGURE 13

PLEC-PTM-6 for the repair of the 1287ins3 mutation  
in plectin deficient patient cells



## FIGURE 14A

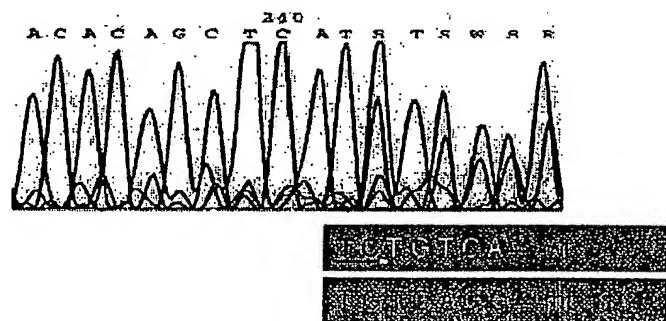
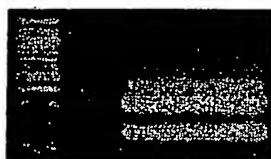
### Trans-splicing Strategy for Col17A1 Gene (GABEB/generalized atrophic benign epidermolysis bullosa)



## FIGURE 14B

# Semi-nested RT-PCR with BPAG2- primer 51-1F, 53-1R and 52-1R

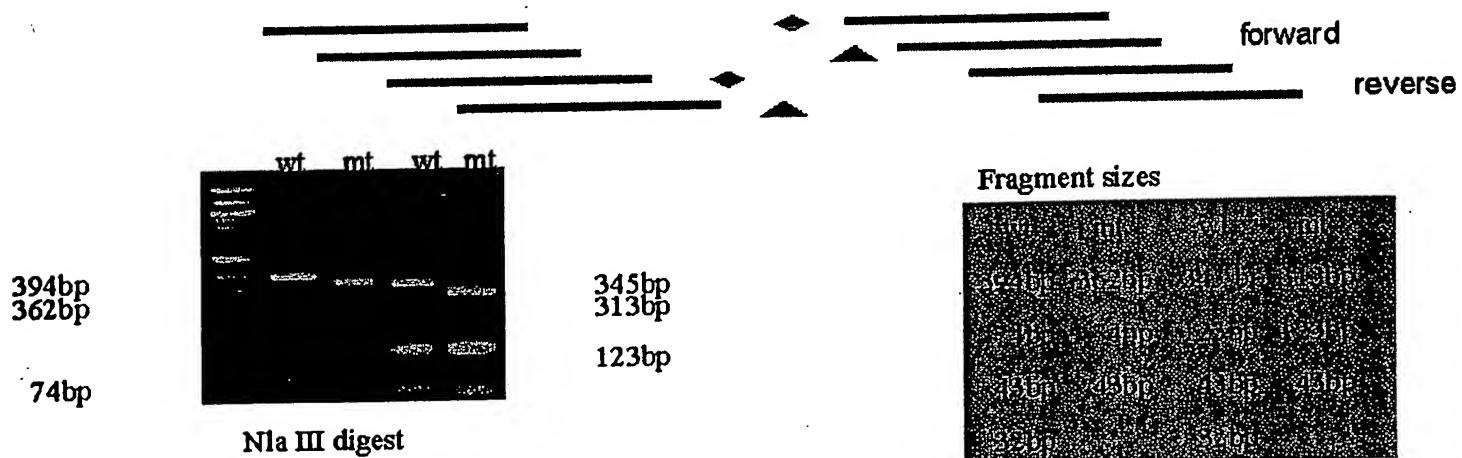
### RT-PCR



F1  
B1  
C

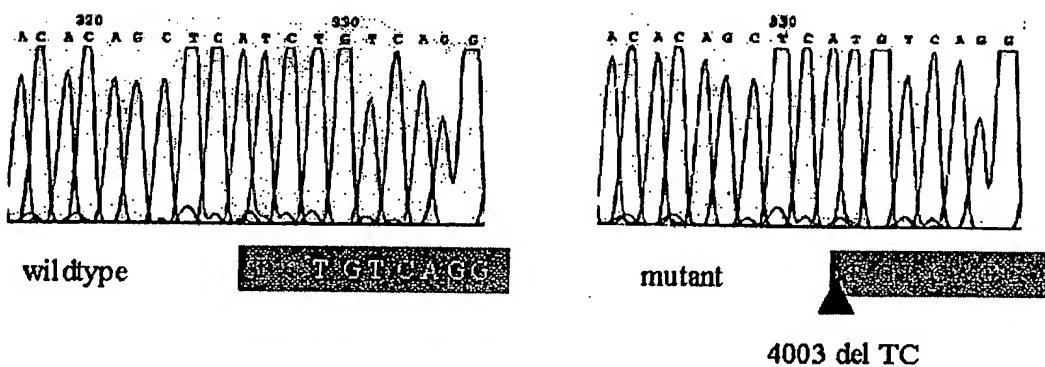
## FIGURE 14C

### TOPO Cloning + Nla III digest



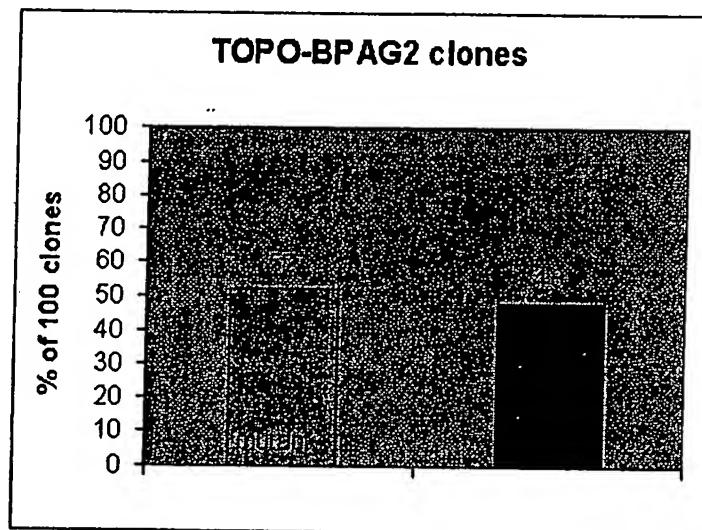
## FIGURE 14D

Sequencing of mutant and wildtype clones confirmed the correct *trans*-splicing of PTM6 into GABEB cells



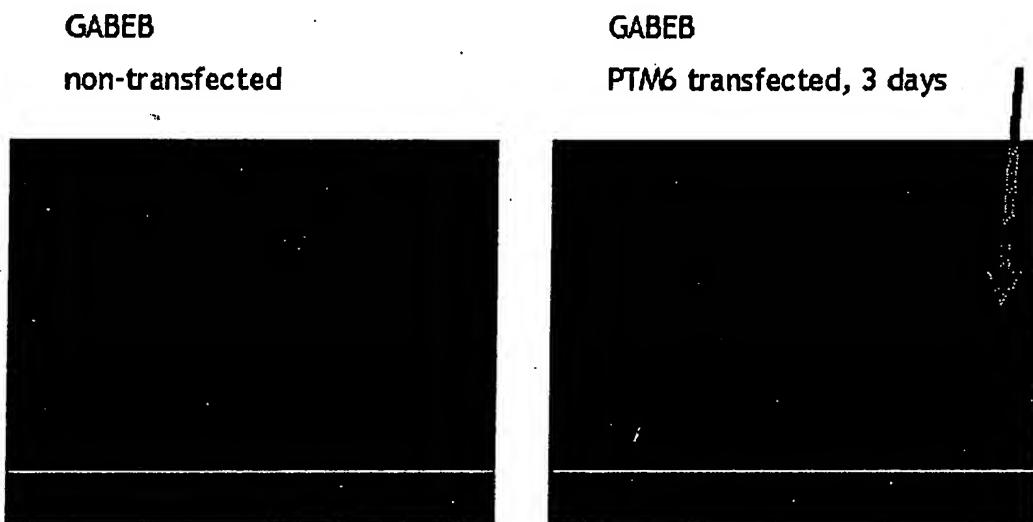
## FIGURE 14E

# Analysis of 100 clones



## FIGURE 15

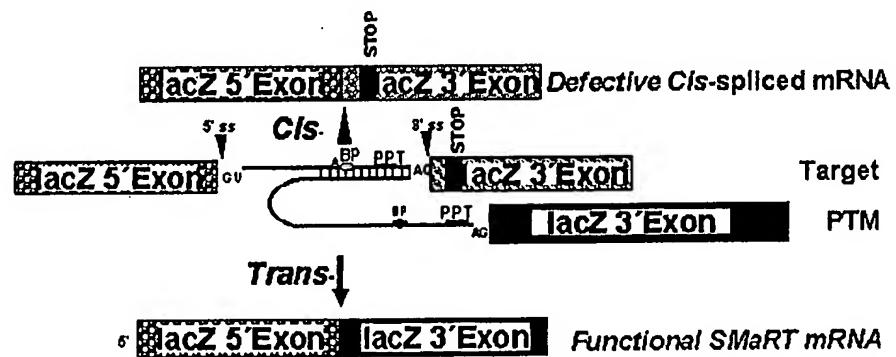
# Immunofluorescence of PTM6- transfected GABEB cells



## FIGURE 16A-B

### Trans-splicing strategy for COL7A1 gene (Dystrophic epidermolysis bullosa)

A.



B.

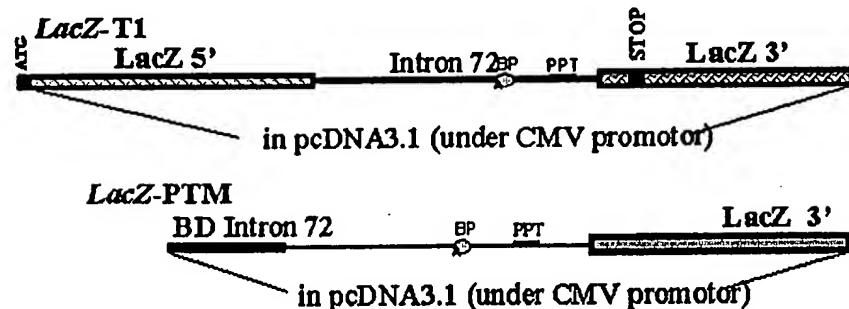


FIGURE 17

$\beta$ -gal Assay of a Cotransfection  
of Col7 Target T1 and PTM 8, 9,  
10 in 293T cells

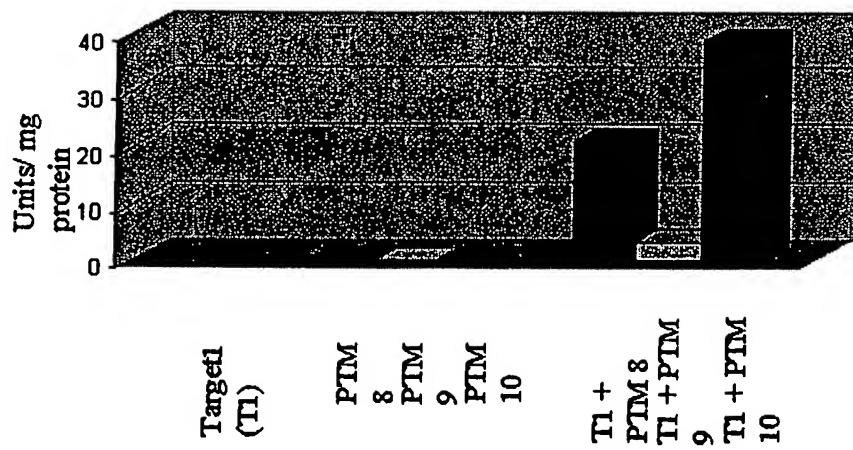
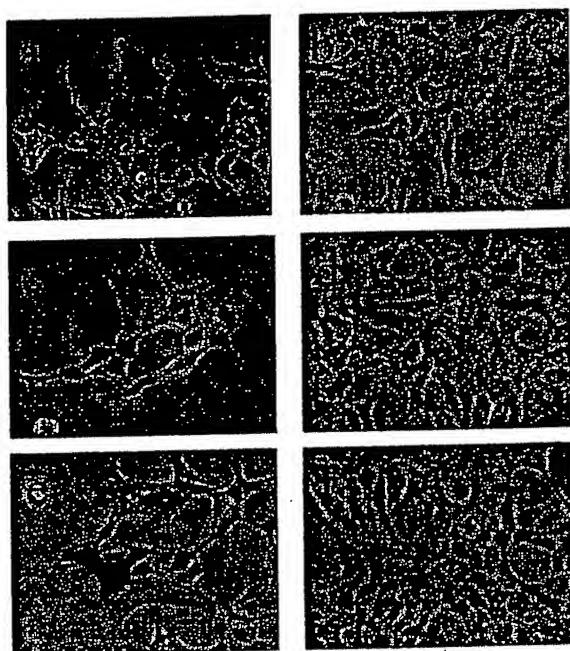


FIGURE 18

$\beta$ -gal staining of Col7 PTM  
transfected human 293T cells



left panel: (a) Target + PTM8, (c) Target + PTM9, (e) Target + PTM10;  
right panel: corresponding control PTM without Target.